
CIRM Planning Grant in Leukemia

Grant Award Details

CIRM Planning Grant in Leukemia

Grant Type: Disease Team Planning

Grant Number: DT1-00709

Investigator:

Name:	Dennis Carson
Institution:	University of California, San Diego
Type:	PI

Award Value: \$52,174

Status: Closed

Grant Application Details

Application Title: CIRM Planning Grant in Leukemia

Public Abstract:

Leukemias are cancers of the blood forming cells that afflict both children and adults. Many drugs have been developed to treat leukemias and related diseases, but in many cases of adult leukemia, the diseases are not curable, and cause disability and eventual death. More than in other cancers, scientists understand the exact molecular changes in the blood forming cells that cause leukemias, but it has been very difficult to translate the scientific results into new and effective treatments. The main difficulty has been the failure of existing agents to eliminate small numbers of leukemia stem cells that persist in patients, despite therapy, and that continue to grow, spread, invade and kill normal cells. In fact, the models used for drug development in the pharmaceutical industry have not been designed to detect drugs or drug combinations capable of destroying the leukemia stem cells. Drugs against leukemia cell stems may already exist, or could be simple to make, but there is not an easy current way to identify them.

Recently, physicians and scientists at universities and research institutes have developed tools to isolate and to analyze leukemia stem cells taken directly from patients. By studying the leukemia stem cells, physicians and scientists have learned what molecules these cells need to survive. We have identified key switches that reprogram normal stem cells into leukemia stem cells. The experimental results strongly suggest that it will be possible to destroy leukemia stem cells with drugs or drug combinations, without harming most normal cells. Now we need to translate the new knowledge into practical treatments.

The goal of the CIRM Planning Grant in Leukemia is to forge a team, and to implement a planning process that will bring together highly experienced scientists and physicians who have developed the leukemia stem cell test systems, with our existing collaborators in the vigorous California pharmaceutical industry, who already have drugs, but lack the cells and model systems to assess their efficacy against leukemia stem cells. The requested funds will support the establishment of an interactive group, a review of projects by expert external advisors, the integration of selected projects according to a practical timeline, and the generation of a reporting structure for CIRM. The new CIRM-supported interactive group will have the resources to introduce into the clinic, within four years, new drugs for leukemias and to change the ways that these diseases are treated for the benefit of the citizens of California.

**Statement of Benefit to
California:**

Thousands of adults and children in California are afflicted with leukemia and related diseases. Although tremendous gains have been made in the treatment of childhood leukemia, 50% of adults diagnosed with leukemia will die of their disease. Current therapies can cost tens of thousands of dollars per year per patient, and do not cure the disease. For the health of the citizens of California, both physical and financial, we need to find a cure for these devastating illnesses.

What has held up progress toward a cure? Compelling evidence indicates that the leukemias are not curable because available drugs do not destroy small numbers of multi-drug resistant leukemia stem cells. The animal models that pharmaceutical companies use for drug testing do not measure the leukemia stem cells, so the companies cannot identify drugs that kill them. Experts in the industry are aware of the problem, but have had difficulties forging alliances with physicians and scientists at academic medical centers who have models for primary human leukemia stem cells needed for drug testing, and systems for assessing efficacy in clinical trials.

The CIRM Planning Grant in Leukemia, if awarded, will provide the initial resources required to implement a new paradigm for drug development, i.e., a focused team approach with participants from academia and industry sharing a common goal, with clear-cut areas of collaboration. The team approach is necessary to find a cure for leukemia, since the current system is inadequate. Since so much scientific knowledge exists about leukemia stem cells, the systems established in the CIRM Disease Specific Planning Grant will also serve as a model for the clinical development of drugs against solid tumor stem cells, which are not yet well understood.

In summary, the benefits to the citizens of California from the CIRM disease specific grant in leukemia are:

- (1) direct benefit to the thousands of leukemia patients
- (2) financial savings due to definitive treatments that eliminate the need for costly maintenance therapies
- (3) new alliances between the academic and pharmaceutical sectors in California that lead to new jobs
- (4) the deployment of a new drug development team approach that will be applicable to other cancers
- (5) Realization of the CIRM mandate to deliver highly effective novel therapies within a short time frame to benefit the health of Californians afflicted with leukemia.

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